European Medicines Agency
Pre-authorisation Evaluation of Medicines for Human Use

Document Date: London, 6 October 2009
Doc.Ref.: EMEA/COMP/157131/2004 Rev.1

Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in March 2009 on request of the Sponsor.

Committee for Orphan Medicinal Products

Public summary of positive opinion for orphan designation of sabarubicin for the treatment of small cell lung cancer

On 21 December 2004, orphan designation (EU/3/04/255) was granted by the European Commission to Menarini Ricerche S.p.A , Italy, for sabarubicin for the treatment of small cell lung cancer.

What is small cell lung cancer?
Small cell lung cancer is a disease in which cancer (malignant) cells develop in the lungs. This type of lung cancer accounts only for about 20% of all lung cancer cases. Usually it develops in the central part of the lungs and the cancer cells are typically small compared with the other types of lung cancer. Signs of cancer are difficult to detect in early stages of the disease, and the majority of the patients are diagnosed when the disease has disseminated and cannot be surgically removed. Small cell lung cancer is a life-threatening disease.

What is the estimated number of patients affected by the condition?
At the time of designation, small cell lung cancer affected approximately 0.5 in 10,000 people in the European Union (EU)[*]. This is equivalent to a total of around 23,000 people, and is below the threshold for orphan designation, which is 5 people in 10,000. This is based on the information provided by the sponsor and knowledge of the Committee for Orphan Medicinal Products (COMP).

What treatments are available?
The main treatment of small cell lung cancer is chemotherapy (i.e drugs that kill cancer cells) and radiotherapy (i.e using high-dose x-rays or other high-energy rays to kill cancer cells).
Several medicinal products were authorised for the treatment of small cell lung cancer, alone or in combination in the Community at the time of submission of the application for orphan designation.
Sabarubicin might be of potential significant benefit for the treatment of small cell lung cancer because it might improve the long-term outcome. This assumption will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status.

How is this medicine expected to work?
Sabarubicin is expected to kill dividing cells since it might interfere with the production of a structure called deoxyribonucleic acid (DNA), the cellular structure which carries the genetic information and which is necessary for the division and survival of both normal and abnormal (i.e. tumour) cells.

[*]Disclaimer: For the purpose of the designation, the number of patients affected by the condition is estimated and assessed based on data from the European Union (EU 25), Norway, Iceland and Lichtenstein. This represents a population of 459,700,000 (Eurostat 2004).

7 Westferry Circus, Canary Wharf, London, E14 4HB, UK
Tel. (44-20) 74 18 84 00 Fax (44-20) 75 23 70 40
E-mail: orphandrugs@emea.europa.eu http://www.emea.europa.eu

What is the stage of development of this medicine?
The effects of sabarubicin were evaluated in experimental models.
At the time of submission of the application for orphan designation, clinical trials in patients with small cell lung cancer were ongoing.

Sabarubicin was not marketed anywhere worldwide for small cell lung cancer or designated as orphan medicinal product elsewhere for this condition, at the time of submission.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 11 November 2004 a positive opinion recommending the grant of the above-mentioned designation.

Opinions on orphan medicinal product designations are based on the following three criteria:

- the seriousness of the condition;
- the existence of alternative methods of diagnosis, prevention or treatment;
- either the rarity of the condition (affecting not more than 5 in 10,000 people in the Community) or insufficient returns on investment.

Designated orphan medicinal products are products that are still under investigation and are considered for orphan designation on the basis of potential activity. An orphan designation is not a marketing authorisation. As a consequence, demonstration of quality, safety and efficacy is necessary before a product can be granted a marketing authorisation.

For more information:
Sponsor's contact details:
Menarini Ricerche S.p.A
Via Tito Speri 10
I-00040 Pomezia (Roma)
Italy
Telephone: +39 055 568 03 13
Telefax: +39 055 56 80 99 54
E-mail: sabarubicin@menarini-ricerche.it

Patients' associations contact points:

Cancer BACUP
3 Bath Place
Rivington Street
London
EC2A 3JR
United Kingdom
Telephone: +44 20 76 96 90 03 / 0808 800 1234 (freephone for UK)
Telefax: +44 20 76 96 90 02

Ligue Nationale Contre le Cancer
13 Av. de la Grande Armee
75116 Paris
France
Telephone: +33 1 45 00 00 17
Tefefax: +33 1 45 00 63 06
E-mail: ligue@ligue-cancer.net

The Association of European Cancer Leagues (ECL)
c/o Belgian Federation against Cancer
Chaussée de Louvain, 479
B-1030 Brussels
Belgium
Telephone: +32 2 743 3705
Telefax: +32 2 734 9250
E-mail: chartmann@cancer.be

Translations of the active ingredient and indication in all EU languages and Norwegian and Icelandic

Language	Active Ingredient	Indication
English	Sabarubicin	Treatment of small cell lung cancer
Czech	Sabarubicin	Léčba malobuněčného karcinomu plic
Danish	Sabarubicin	Behandling af småcellet lungecancer
Dutch	Sabarubicine	Behandeling van kleincellig longcarcinoom
Estonian	Sabarubitsiin	Väikeserakulise kopsuvähi ravi.
Finnish	Sabarubisiini	Keuhkojen pienisolusyövän hoito
French	Sabarubicine	Traitement du cancer du poumon à petites cellules
German	Sabarubicin	Behandlung des kleinzelligen Lungenkarzinoms
Greek	Σαβαρουβικίνη	Θεραπεία του μικροκυτταρικού καρκίνου του πνεύμονα
Hungarian	Sabarubicin	Kissejtes tüdőrák kezelése
Italian	Sabarubicina	Trattamento del cancro al polmone a piccole cellule
Latvian	Sabarubicīns	Sīkšūnu plaušu vēža ārstēšana
Lithuanian	Sabarubicinas	Smulkialąstelinio plaučių vėžio gydymas
Polish	Sabarubicyna	Leczenie raka drobnokomórkowego pluc
Portuguese	Sabarrubicina	Tratamento do carcinoma de pequenas células do pulmão
Slovak	Sabarubicín	Liečba malobunkového karcinómu pľúc
Slovenian	Sabarubicin	Zdravljenje drobnocelicnega raka pljuc
Spanish	Sabarubicina	Carcinoma de pulmón de células pequeñas
Swedish	Sabarubicin	Behandling av småcellig lungcancer
Norwegian	Sabarubicin	Behandling av småcellet lungekreft
Icelandic	Sabarúbicín	Til meðferðar við lungnakrabbameini af smáfrumugerð